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Neutropenia Support Assoc. Inc.

P.O. Box 243, 905 Corydon Ave. Winnipeg, Manitoba R3M 3S7

Canada Falls behind The World

G-CSF (Neupogen) is approved for use in United Kingdom, Luxembourg, Ireland, Greece, Germany, France, Denmark, Portugal, Netherlands, Spain, United States, France, and Italy to name a few. Pending approval in Canada, Belgium and Australia. The delays due to our Canadian HPB "political problems" is incomprehensible. We suspect the cost benefit analysis ie: dollar value of lives saved is the root of the problem. Documentation shows hospital stays are significantly reduced, treatment therapy greatly enhanced and complications lessened. The delays do not make sense from any point of view.

Troubled

Words from Dr. Jon M. Gerrard

I write to you because I am concerned about what appears to be undue delays in the licensing and availability of a new drug called granulocyte macrophage colony stimulating factor often called just GM-CSF. As you are aware, both GM-CSF and its relative granulocyte-colony stimulating factor (G-CSF) have been shown in clinical trials to be very useful for decreasing the extent of neutropenia and the incidence of infection in children with cancer receiving extensive chemotherapy or bone marrow transplantation. We were informed at the end of last year that the application for GM-CSF had been submitted to the Health Protection Branch and approval was anticipated in March, 1991. There was delay after delay and finally this Fall we were told that the individuals who had been responsible for looking after the licensing of GM-CSF at the Health Protection Branch had been entirely replaced and the study of this drug and its potential approval for licensure was beginning all over again at step zero. This seems, from this distance, to be a very disorganized state of affairs at the Health Protection Branch in the assessment and evaluation of what is potentially a very new and important therapeutic drug. There has been no adequate explanation for this delay.

Message from the Treasurer

We thank all those continuing to support in its earliest stages, the development here of a neutrophil function laboratory run by Dr. Bonnie Cham. Also through your support we continue to increase the awareness and understanding of neutropenia caused congenitally and as the result of the Chemotherapy that is given to those with cancer. Regarding Dr. Jon Gerrard's letter, we shall continue to focus primarily on this issue in 1992. Our secondary project shall hopefully be a **Spring Fling Fashion Fundraiser.** We are in the initial stages of planning and welcome your assistance. Please contact **Lorna Stevens 477-0540.** We are delighted with the response from our efforts in reaching out through this news letter to the public and the medical community at large. We have to date donated $11,540.00 for our educational and research projects. **We need your continued support.** Tax deductible receipts will be issued. So Renew your annual membership now:

Individual and family	$10.00
Business or Corporate	$25.00

Please send your cheques to:

Neutropenia Support Association Inc.
P.O. Box 243 905 Corydon Ave.
Winnipeg, Manitoba
R3M 3S7

FDA approves drug to help cancer patients

Treatment boosts white cell count

The Associated Press

WASHINGTON - The Food and Drug Administration approved on Thursday a new, genetically engineered drug it said could annually help an estimated 225,000 cancer patients fight life-threatening infections while on chemotherapy. The drug works by boosting production of infection-fighting white blood cells, which are reduced or killed entirely by many kinds of cancer-fighting drugs. FDA Commissioner David Kessler said the drug, called granulocyte colony-stimulating factor, or G-CSF, "is a pioneer therapeutic product." While other biotechnological treatments have proved useful for only small numbers of patients, he said, a large number of cancer patients can benefit from G-CSF. The drug may be used for patients undergoing myelosuppressive chemotherapy, a type that destroys cancer cells and certain immune cells. These patients, an estimated 225,000 each, are vulnerable to infections that can be life-threatening, he said. G-CSF does not affect the cancer itself, the FDA said. But it can lessen the chances that a patient will get an infection, reducing the need for antibiotics and hospitalization, This results "in a significant improvement in the quality of life for these patients," the FDA said. In clinical studies in more than 350 patients with different types of cancer, no serious side effects were reported, the FDA said. The most common reaction, reported by 20 percent of the patients, was mild to moderate bone pain, which was relieved in most patients with acetaminophen, the FDA said. Acetaminophen is the principal ingredient in Tylenol. G-CSF is one of a group of proteins called colony stimulating factors, which are found only in tiny amounts in human tissue but can be mass-produced with gene-splicing techniques.

News Letter gets Financial Backing

In response to your request for funding for the Neutropenia Support Association newsletter, I am pleased to enclose a cheque for $1,000.00. Please note that since **Schering Canada and Sandoz Canada** are jointly developing GM-CSF, please be sure to mention both companies in any acknowledgments. We are very pleased to be able to contribute to the success of your newsletter.

First News Letter Brings Results

I just became aware of the existence of your Neutropenia Support Association Incorporated. I would like to explore with you the possibility of funding for our research on congenital and acquired forms of neutropenia in children, as well as a variety of other marrow failure disorders.

Melvin H. Freedman, MD
Head, Clinical Hematology
Professor, Dept. of Pediatrics
The Hospital For Sick Children

We Welcome a New Family

I received a copy of your Vol. 1, No. 1 Neutropenia Newsletter yesterday, forwarded to me by Dr. L.L. deVeber, Director Pediatric Haematology/Oncology and Professor Pediatrics at the University of Western Ontario. It is with extreme interest that I am sending this letter. ..... I gather from your newsletter that your group is the only Neutropenia Support Group in existence. I would appreciate as much information on neutropenia that you could provide me with as well as information on this new drug being developed for neutropenia .... I am also very grateful that there is actually research going on to discover more about this rare blood disorder, and hopefully a cure. I thank you in advance for any information you can provide.

Yours sincerely,
Cathy Riley

Winnipeg gets a Research Lab

Dr. Bonnie Cham , is in the process of setting up a neutrophil function laboratory and developing a neutrophil research program. This is a very important project for Manitoba because we have never had an individual before whose primary focus was understanding neutrophils and with considerable interest in taking care of patients with congenital neutropenia and other neutrophil function disorders. These conditions lead to infections which are frequent, and I suspect because we haven't had somebody here with this research interest, that we may be missing a number of children who have neutrophil function problems.

UpDate from Dr. Bonnie Cham

I would like to begin this letter by expressing my gratitude to your organization for the research funds which you have provided to Dr. Gerrard for investigation of neutrophil function and dysfunction. We moved into our lab space in January, 1991 and the work is beginning to progress. As some of you will recall, one of the research projects which we are conducting involves the effects of flax oil administration on psoriasis. Psoriasis is a skin condition characterized by inflammation and neutrophils appear to be important in its development. By altering the type of fatty acids available to the neutrophil, we hope to make them less pro-inflammatory. When we discussed this at the group meeting in September, several members mentioned that they had relatives with psoriasis. We are therefore interested in obtaining family histories from patients with neutropenia to determine whether there is a higher incidence of psoriasis in these families. I would like to arrange to interview patients with congenital neutropenia, or a patient if appropriate, to obtain detailed family histories. The interview would take approximately thirty minutes, and would be arranged at a mutually convenient time. The areas I would be inquiring about would include the occurrence of neutropenia or other blood disorders, psoriasis and other skin disorders, arthritis, and the presence of any known inherited disorders. We would like to obtain information about the extended families of both parents and include grandparents, parents, aunts, uncles, cousins, and children of patients with neutropenia. If you would be interested in participating in this type of study, please contact me at the Cancer Foundation 787-4108. I will be in touch with you to arrange an interview time. The other areas of interest is whether we can identify people who are carriers of congenital neutropenia. As an initial approach to this problem, we would like to measure super oxide production of neutrophils from parents of patients with congenital neutropenia before and after incubation of the neutrophil G-CSF and /or G-CSF. This would involve taking 30 cc of blood (approximately 6 tbsp. or 1.0 oz) on the morning of the experiment. The only potential side effect would be a bruise at the site of the needle poke and mild discomfort. Again, if you would be willing to participate, please contact me. If you have any questions about this, I can be reached at the Cancer Foundation at 787-4108. Thank you for your support and co-operation.

More updates from Dr. Cham

When Lorna Stevens mentioned that another issue of the newsletter was coming out. I agreed with her that it would be a good opportunity to relay some of our research activity of the last year. As many of you are aware, I have now been in Winnipeg for just over one year and conducting research in to various aspects of neutrophil function under the supervision of Dr.Jon Gerrard. My primary focus over the past year has been to characterize the location of a protein, located in granules of neutrophils, which Dr. Gerrard originally identified in platelets. This has been moving along fairly well and we are now attempting to define the role of this protein in neutrophil function. Another project we are involved with is to attempt to delineate the possible role of histamine within the neutrophil. This work is being carried out by a graduate student in the lab, Esther Genaske, and again we are making progress. Of more interest to this group, is a project I hope to complete over the next two months looking at parents of patients with congenital neutropenia. As most of you are aware, the hormone GM-CSF and G-CSF have shown promise in the treatment of patients severely affected with neutropenia, and it is possible that the underlying defect in these disorders may be related to the way these patients normally make or respond to these hormones. As a result, we thought it would be interesting and informative to test neutrophils from parents of patients with neutropenia to see if parents, who may be carriers of the genetic change leading to neutropenia, have either augmented or diminished responses to these hormones. I appreciate the response I received from the neutropenia families when I sent out a letter asking for volunteers in June, and I will be in touch with you shortly to arrange time for the testing to be done. Once again, thank you for your on going support in our research endeavors.

As you are aware, we are continuing with research in to neutrophil physiology in the lab of Dr. Gerrard. We appreciate the support your group has been able to offer us in the past and are returning to you once again to request further financial support. As you are aware, one of the projects presently being conducted in the lab is looking at the potential role of histamine in neutrophil function. We have been looking at this by examining the ability of various anti-histamines to block neutrophil function. We would like to examine this directly by adding histamine to the neutrophils in order to more directly assess the effect of histamine. However, we are studying an effect of histamine within the cell and histamine itself does not cross the cell membrane well enough. In other cells this problem has been circumvented by permeabilizing (or making holes in) the cell membrane in order to allow histamine to enter the cells. The most reliable way is electropermeabilization, which is used very successfully in many labs. This **requires a piece of equipment which would cost approximately $3500 including tax.** A second project we are involved with has been looking at the location of ''granulophysin'', a granule membrane protein, in resting and activated neutrophils. This project has involved using the FACS machine (Fluorescent activated cell sorter) which is located in the Department of Immunology. **We would request a further $1000 to assist towards ongoing costs of reagents and technical time for using the FACS machine.** We appreciate any assistance your group can offer us towards these goals.

Dr. Gerrard stresses need for Library

With congenital neutropenia, as with a number of other rather rare disorders, the general knowledge of this condition even among medical and nursing staff because of its rarity, is not as good as it should be. One of the important steps in increasing awareness and understanding of congenital neutropenia and other rare blood disorders, is for expanded information on these conditions to be available in the library at the Children's Hospital. I see this as not only important for medical and nursing staff, but also for other hospital people, including social workers, occupational therapists, etc. who have to deal with these children. I think it is also important for families that there be some generally available in-depth library material on these conditions.

And the library was Born

On behalf of the Department of Pediatrics and Child Health and its section of Pediatric Hematology, I would like to thank you and the Neutropenia Support Association for its generous contribution to the Pediatric Hematology Library. We are always grateful to specific support groups who feel that it is important to support our programs in this manner. Thank you once again for your wonderful donation, and if I can be of assistance to you at any time, please let me know.

Agnes J. Bishop, MD, FRCPC, LLD (Hon)
Professor and Head
Dept. of Pediatrics and Child Health

Further to our several telephone conversations over the past few months, the list attached identifies titles already purchased and those we will order, to enhance educational material available in our library on Neutropenia and other rare blood disorders. I would like to thank you and your association once again, for providing resources for our library to obtain these materials. At present we believe the funds you donated last fall will be sufficient to cover the costs of acquiring these educational materials. Should there be any funds remaining we will use them to acquire other materials - journal articles or text books - as they are published. I would suggest that there are other educational needs with regard to rare blood disorders such as Neutropenia which your group may wish to support. Educational videos or puppets for use in educating children about their illnesses and treatments are two ideas you may wish to consider. Please contact me if I can be of further assistance.

Books For The Library

Titles already Purchased

Blood: Textbook of Hematology, J.H. Jandel

Hematology, W. J. Williams.

Hematopoietic Growth Factors in Clinical Applications, R. Mertelsman.

Titles To Be Purchased

Hematologic Problems in the Newborn, Oski, Naiman, Saunders, Phil, 1982 or 1984

Two back issues of Hematology Oncology Clinics of North America
Volume 2 No. 1 March 1988
Volume 2 No. 2 June

Iron Nutrition in Infancy and Childhood, Steckel, A., New York, Raven Press 1984

Molecular Basis of Blood Diseases, Nienhuis, A.W. (eds) W.B. Saunders, 1986

The Clinical Approach to Thalassimia, Modell B., New York, Grune & Stratton, 1984

Hematopoietic Stemtells, Golde, D.W., Takaku F., (eds), New York, Maral Depper, 1985

Hemostasis and Thrombosis, Basic Principles and Clinical Practice, Colman, Hirgh, Marden, Salzar, J.B. Lipincott Co., 1987

Biology of Platelets, Phillips D.R & Shuman, M.A. (eds), San Diego Academic Press.

20 Canadian Doctors Visit Amgen Plant

From Winnipeg, Dr. Eric Bow, Dr. M Rubinger, and Dr. Jacek Winiarski, visited Los Angeles. Reports presented by Dr. Laurence Boxer and others concluded, Chromosome #17 was identified as deficient of hormone, the hormone, the size of a protein kilodolton with the molecular weight much smaller than an antibody, and the G-CSF (Neupogen) hormone binds to a receptor on the surface of the neutrophil which activates it and starts the nucleus to divide. The reports on the studies of severe neutropenia patents were overwhelmingly successful, except for 3 reported cases. Neupogen will be used with treatment of: chemotherapy neutropenias, low myeloid malignancies, bone marrow transplant, chronic neutropenias, priming for chemotherapy, aids patients suppressed on AZT, short term therapy for aplastic anemia, pre-leukemic disorders-myelodysplasia, burns, neonatal septicemia and as an adjunct to antibiotic treatment.

Letter From Joann Primrose

Our daughter Jaclyn was 8 months old when it seemed she was always getting sick. It was one ear infection after another or a cold with a high temperature that would last for two-three weeks. My husband and I, being first time parents, couldn't help but wonder what we were doing wrong in caring for our daughter. I always felt there was something seriously wrong with Jaclyn, however our pediatrician assured us that Jackie was just a normal, healthy child. She stated that children are frequently ill during the first year of their life. After a number of infections I decided to take Jackie to another pediatrician for a second opinion. He also felt that it was quite normal for an infant to be sick as often as Jaclyn had been. He did, however, decide to take a blood sample from Jaclyn and discovered that Jackie's neutrophil count (a type of white blood cell that fights off bacterial infections) was near zero. He felt at that time that her neutrophils may have been wiped out from many viruses Jaclyn had developed in the past few months and that over time her count would return to normal. We took Jaclyn for a blood test every week but her count did not improve .Jaclyn was constantly sick. Approximately two months had passed when Jaclyn's pediatrician contacted me at work to say that the latest blood test showed Jaclyn's neutrophils had not increased. He informed me that we would wait one more month and if there was still no change in Jackie's neutrophil count he would refer her to a specialist at the Manitoba Cancer Research Building. He also stated he was concerned that Jaclyn may have something seriously wrong with her and that leukemia was even a possibility. Being faced with the thought of leukemia, we certainly did not want to wait another month to see if Jackie's neutrophil count would improve. I felt that we had wasted enough time already. My boss recommended his children's pediatrician, Dr. Esquivel, who worked out of the Winnipeg Clinic. He said he was an excellent doctor and felt that Dr. Esquivel would be able to help Jaclyn. I made an appointment with Dr.Esquivel the very next day. He immediately diagnosed Jaclyn as having a rare blood disorder called neutropenia. He referred us to Dr. Sara Israels of the Winnipeg Cancer Research Foundation. She performed a bone marrow test on Jaclyn and confirmed Dr. Esquivel's diagnosis. The bone marrow test showed that Jaclyn's bone marrow produced neutrophils but they did not mature enough to enter Jackie's blood system. The bone marrow test also ruled out all other serious possibilities, i.e. leukemia, liver disease, etc. My husband and I felt better once we finally knew what was wrong with our daughter, and knowing it was not in the nurturing of our child. I am happy to report that Jaclyn's condition has significantly improved over the past year. Her neutrophil count has increased from approximately 250 to over 1,000. It is amazing what a difference the extra neutrophils have made in Jaclyn's health. She no longer requires antibiotics every ten days or so. Jaclyn is now off antibiotics anywhere from 6-8 weeks before she requires medicine. She has not had an ear infection for well over a year. In closing, I would like to urge all parents who may feel their children are too frequently sick, to have them tested for neutropenia. It is very important that these children are diagnosed immediately as simple childhood viruses that are not treated with antibiotics may develop into something more serious.

Use This Handy Form To Send In Your Tax Deductible Donations

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Send to Neutropenia Support Assoc. Inc. P.O. Box 243, 905 Corydon Ave. Winnipeg, Manitoba R3M 3S7